Patient Innovation:

its prevalence, antecedents and impact

Matilde Czernin

Supervisor: Prof. Pedro Oliveira

Co-supervisor: Leid Zejnilovic

Dissertation submitted in partial fulfilment of requirements for the degree of MSc in Business Administration, at Universidade Católica Portuguesa,

September 2013

Note: This research was funded by the Portuguese Foundation for the Science and Technology (FCT) and the CMU Portugal Program through project CMU-PT/OUT/0014/2009. The survey development and data collection was conducted in the context of a project funded by the Peter Pribilla Foundation (Germany).

Acknowledgements

I would like to express my sincere gratitude to my supervisor Professor Pedro Oliveira for all the encouragement given throughout this thesis. I am deeply thankful for the opportunity to work with such rich data and interesting research project. Moreover, I am very grateful to my co-supervisor Leid Zejnilovic for all the tireless support and fruitful discussions.

I would also like to make a very special acknowledgement to Vitor for the essential technical help with the statistical modelling component of this work. Finally, I would like to thank the one person without whom I would not had been able to do this thesis, João.

Abstract

In order to maintain the level of healthcare that we now associate with developed countries, innovation is imperative. Previous literature has shown that patients of chronic diseases are often involved in the development of new treatments and medical devices to help them cope with their health-condition. However the innovation developed by patients is often ignored or even rejected. A possible approach to address this issue is to open up healthcare innovation, by allowing patients and their caregivers to become themselves active contributors to the innovation process. In this context, the aim of this dissertation is to (1) quantify the extent to which patients and caregivers of rare diseases innovate, (2) find demographic and non-demographic antecedents of patient innovation, and (3) assess how the innovations impact the lives of patients.

A telephone survey was conducted with the main objective of measuring the extent to which respondents had innovated, or not. 496 patients and caregivers of 250 rare diseases responded to the survey. Following the data collection we performed a descriptive analysis of the data and a multiple logistic regression to identify statistically relevant predictor variables of patient innovators.

We found that 13% of respondents had innovated, and the variables that emerged as predictors of being an innovator are: higher level of education, being unemployed or looking after at home, being aware of the expenses with the disease, and Information and Communication Technology readiness. On the other side, being single has a negative impact on the propensity to innovate when compared with being married. Moreover, in a 7-point Likert scale measuring the quality of life of the patient, the innovations led to an average improvement of 2.4 points.

Not only are patients developing completely new-to-the-market innovations, that are improving the patients' quality of life, but they also assume the risks of trying solutions that had not yet been tested. This study suggests that the current producer-based and paternalistic healthcare model should be revised, so patients are given the chance of playing a more proactive role.

Key words: user innovation; patient innovation; antecedents of innovation; healthcare innovation

Resumo

De forma a mantermos a saúde nos níveis que hoje associamos com países desenvolvidos, a inovação é imperativa. Estudos anteriores revelaram que em vários casos pacientes de doenças crónicas estão envolvidos no desenvolvimento de novos tratamentos e equipamentos médicos que os ajudam a lidar com a sua condição. Contudo, as inovações desenvolvidas por pacientes são frequentemente ignoradas ou até rejeitadas. Uma possível solução para superar esta crise de inovação seria deixar que pacientes e cuidadores se tornassem eles próprios contribuidores ativos no processo de inovação. Deste modo o objectivo desta dissertação é (1) quantificar até que ponto pacientes e cuidadores de doenças raras inovam, (2) identificar antecedentes demográficos e não-demográficos de inovação por pacientes, e (3) avaliar o modo como as inovações por pacientes afetam a vida dos pacientes.

Conduziu-se um questionário telefónico com o fim de determinar até que ponto os entrevistados teriam inovado ou não. 496 pacientes e cuidadores de 250 doenças raras responderam ao questionário. Após a recolha de dados, efetuámos uma análise descritiva dos dados bem como uma regressão logística múltipla de forma a identificar variáveis estatisticamente relevantes preditoras do fenómeno de inovação por pacientes.

Constatámos que 13% dos respondentes inovaram. As variáveis que emergiram como preditoras foram: educação superior, estar desempregado ou ser doméstico, estar ciente das despesas com a doença, utilização de tecnologias de informação. Por outro lado, ser solteiro, quando comparado com ser casado, tem um impacto negativo na propensão para inovar. Adicionalmente, numa escala de Likert de sete pontos que mediu a qualidade de vida do paciente, denotou-se uma melhoria média de 2.4 pontos após a inovação.

Estes indivíduos não apenas desenvolvem inovações que melhoram a qualidade de vida dos pacientes, mas assumem também o risco de experimentar soluções que não foram ainda testadas. Este estudo sugere que o atual modelo de saúde paternalista, cujo epicentro são os produtores, deverá ser revisto tendo em vista a possibilidade de pacientes assumirem um papel mais proactivo.

Palavras chave: inovação por utilizadores; inovação por pacientes; antedecentes de inovação; inovação na saúde

Contents

List of Figures

List of Tables

Chapter 1

Introduction

Health expenditure in OECD countries has grown at an average rate of 5% from 2000 to 2009 (OECD, 2013a). The GDP growth rate is less than half for the same period. The result: health expenditure was responsible for an average slice of 9.5% of OECD members' GDP in 2011. Demand and expectations regarding healthcare have been increasing astoundingly, yet in order to maintain the level of healthcare that we now associate with developed countries enormous innovation is imperative. This task is far from easy to accomplish. In the current healthcare system, healthcare innovation is an increasingly lengthy and costly process. The average cost per new drug development to the point of marketing approval was estimated on 802 million US$ (DiMasi et al., 2003). Bureaucracy is not the least of worries, only to activate phase III trials 296 different process steps are required (Dilts et al., 2009), thus, not surprisingly, the average time for clinical and approval phases is 8.1 years (Kaitin and DiMasi, 2010).

In this context, it is important to think out of the box, and explore new forms of innovating. Healthcare users, such as patients and their caregivers, due to their unique knowledge about the diseases, can potentially become very powerful sources of innovation. In point of fact research has already shown very successful innovation cases by patients and their families (Oliveira et al., 2011; Oliveira, 2012; Habicht et al., 2013). We build upon the user-innovation definition by von Hippel (2007), where patients are users of health-care services. In that sense, patient innovators (PI) are individuals (patients or caregivers) who have developed a new equipment, medical device, treatment, therapy, strategy, habit or behavior with the intention of using (as opposed to sell) to treat or better cope with their health condition (Shcherbatiuk, 2012).

The main objective of this dissertation is to empirically analyze the extent of patient innovation as well as to identify its antecedents and evaluate its importance or impact. In order to approach our problem statement, we will explore three main research questions:

- We first need to know the extent to which patients are innovating: ***To what extent are patients and their families innovating?***
- Moreover, when it comes to a serious matter such as health, it is only logical to encounter more conservative attitudes. In this sense, society needs to be convinced of the utility of patient innovation, and that is why its impact should be measured: ***What is the impact of patient innovation?***
- Finally, if in fact the phenomenon is substantial and in order to benefit from their advantages, there must be ways of identifying actual or potential patient innovators, so the system can properly incorporate them: ***What factors contribute or withhold patient innovation?***

According to the European Organization for Rare Diseases, a disease is considered rare if it affects less than five in 10,000 persons. A total of 5,000 to 7,000 rare diseases have been estimated, and they affect 6% to 8% of Europe's population (EURORDIS, 2005). We have chosen to study the case of rare diseases in particular because although there has been a growing interest and effort to improve it, investigation for treatments in rare diseases is still scarce and is a very challenging matter for researchers (Griggs et al., 2009). In such an underserved market we expect a significant existence of patient innovation.

Through our contribution, we hope to emphasize the potential patient innovation has to offer, and bring it to the attention of policymakers, regulators and healthcare professionals.

The following sections include a Literature Review (Chapter 2) with the scope of not only positioning our research but also to present the main findings on this area up to date. We will then proceed to determine our set of hypothesis as well as describe in detail the data and methods used to address our research problem and questions in Chapter 3 – Methodology. The results of our work will be presented in detail in Chapter 4. Lastly, we will make a final comment on our results and highlight the limitations and future research directions of our study in Chapter 5.

Chapter 2

Literature Review

In the Literature Review chapter we cover three main topics required to fully contextualize this thesis. Section 2.1 discusses innovation, including its drivers (2.1.1) and what makes it adopted (2.1.2). In section 2.2 user innovation will be presented, including its quantification in different domains, its determinants, and importance. The subsection 2.2.1 covers the case of patient innovation and its potential. Finally in the last section, 2.3, we will analyze the current healthcare model, including an overall picture of healthcare innovation and the roles of the players involved in this industry.

2.1 Innovation

There are many suggested definitions for innovation. It comprises something new, original or different that meets a market or society need (Frankelius, 2009), or simply a new way of doing something (Porter, 1990). In a relatively popular definition, West (1990) defines innovation as "the intentional introduction and application within a role, group, or organization, of ideas, processes, products or procedures, new to the relevant unit of adoption, designed to significantly benefit the individual, the group, or wider society". However, according to Baregheh et al. (2009) the existing definitions are in accordance with the different disciplines, thus he proposed the following integrative definition of organizational innovation 'the multistage process whereby organizations transform ideas into improved products, services, or processes in order to advance, compete, and differentiate themselves successfully in their marketplace". Without disagreement, the most important requirements of an innovation are novelty, having an application component and, naturally, having an intended benefit (Länsisalmi et al., 2006).

Importance of innovation

The importance of innovation for competitiveness, effectiveness and long term economic growth of nations and organizations is unquestionably evident (Drucker, 1985; Porter, 1990; Wolfe, 1994), especially nowadays with increasingly international and globalized competition (Schilling, 2005). In fact, Porter (1990) argued that in order to compete and to sometimes gain competitive advantage, an investment in the creation and dissemination of knowledge is essential.

For firms, innovation holds unquestionable importance, a study by Nås and Leppãlahti (1997) revealed that that non-innovating companies report lower profits, and that these differences are persistent in the future. Hult et al. (2004) confirmed that, independently of the market instability, innovativeness, which is the capability of a firm to engage in innovation, is a key determinant of business performance.

2.1.1 Drivers of innovation

There has always been a great deal of interest in understanding the determinants of innovation, which has not always been a subject of agreement. An important aspect to bear in mind when performing such analysis, is that the best way to address effects of innovativeness and its antecedents is not through an unrealistic analysis of bivariate relationships, but rather through a more integrated approach (Hult et al., 2004). In one of the first studies about the drivers of innovation, Schmookler (1966) concludes that the opportunities with more expected value will have a greater degree of innovation, thus being common to exploit and allocate inventive effort to areas of higher expected demand or profit expectations.

Focusing on organizational and individual level of innovation, Damanpour (1991) performed a meta-analysis to test the relationships between innovation and thirteen of its potential determinants as well as whether the assumption of instability in the results accomplished so far was valid, as there was substantial variance among studies. His study resulted in ten statistically significant relations. Centralization, by disfavoring an environment of participation and communication, has a negative impact on innovation. Contrarily, the following proved to have a positive effect on innovation: specialization, functional differentiation, professionalism, managerial attitude toward change, technical knowledge resources, administrative intensity, slack resources, and external communication and internal communication. Moreover the findings of his study suggested that these relations were stable, and that the instability and variance of previous studies was due to sampling error. Other boosters and key drivers of innovation at a company and individual level are employees with a higher level of experience, or holding a university degree (Rao et al., 2002).

Sometimes innovation comes from sudden and spontaneous flashes in the minds of innovators. These cases are rare though, and most innovation is intentional and comes from determined and focused research of unexpected occurrences, incongruities, process needs, and industry or market changes (Drucker, 1985). Moreover, there is the concept of lead users, that are defined as individuals that experience needs ahead of the market and expect to gain relatively high benefits by developing something to cope with that need, thus being very prone to innovate (von Hippel, 1986; Herstatt and von Hippel, 1992). Likewise, Morrison et al. (2000) argues innovation takes place among these so called lead users, as long as it is cheaper to invent rather than search for a possible existing solution.

2.1.2 Innovation adoption

It is very difficult to tell what makes an innovation prosperous, i.e. adopted. There has always been an enormous effort in research to determine, standardize and predict successful innovations, (Quinn, 1985; Rothwell, 1992; Denning and Dunham, 2010). As Drucker (1985) debated "Innovation requires knowledge, ingenuity, and, above all else, focus", and more important than doing, the work of knowing is what really matters. Porter (1990) defended that pressure, necessity and adversity will more likely lead an innovation to success than the simple hope of gain. Nonetheless, the recommendations we find in the literature not only diverge, but lead to contradictory directions (Denning, 2012).

The numbers speak for themselves, there are thirty thousand new products every year, and only 10% of them succeed. On a business-like point of view, despite what seems natural to assume, there is no relationship between the spending on Research and Development (R&D) and financial performance of firms (Jaruzelski and Dehoff, 2008), as what counts is not how much to spend but how to spend (Kandybin, 2009). According to Christensen et al. (2005) one of the problems is the excessive efforts put on market segmentation according to stereotyped customers and their needs, that result in "products that don't meet real people's needs". He suggests a more practical approach of rather identifying the jobs people need to accomplish, and sell them in an improved way as a product or service. Levitt (2002) argued that in a company very creative ideas will be mere deadweight if they are not implementable. In the Information and Communication Technologies (ICT) field, successful innovation depends on three basic points: a need, knowledge, and favorable economics (Kalmanek, 2012).

Adoption and acceptance behaviour

When assessing the adoption and diffusion of an innovation, on the one hand we can analyze the perceived characteristics of an innovation that include: relative advantage,

complexity, compatibility, trialability, and observability, and on the other hand we have seven aspects to consider regarding the adopters: General Psychological Antecedents, Context-Specific Psychological Antecedents; Meaning, The Adoption Decision, Concerns in Pre-adoption Stage, Concerns during Early Use, Concerns in Established Users (Rogers, 2010). Agarwal and Prasad (1997) found that the perceived innovation characteristics of visibility, compatibility and triability, together with external pressure are relevant when explaining the acceptance behavior and usage of an innovation. However, the author concludes that the variance in the likelihood of continued future use is only explained by relative advantage and demonstrability.

One thing is for sure, in order to maximize society's value through the innovation process, there must be constant dialog between the innovators and the stakeholder groups, including the non-traditional ones (Dormann et al., 2002). In this line of thought, Henry Chesbrough developed the concept of open innovation that he defined as "a paradigm that assumes that firms can and should use external ideas as well as internal ideas, and internal and external paths to market, as the firms look to advance their technology" (Chesbrough, 2003, 2006)

2.2 User innovation

As mentioned in the previous section, for an innovation to succeed there must be a clear link between the idea itself and the needs of those who will use the product or service. That is why 60 to 80 % of important innovations in many industries were a response to environmental factors, i.e. market needs, as opposed to technical opportunities (Utterback, 1974). Inevitably, not everybody sees their needs being met by manufacturers and firms. There are many reasons behind unsatisfied consumer needs. For instance, firms generally concentrate on the average consumer needs, rather than focusing on the exceptions, which many times are indeed as extensive as the rule, leaving them underserved. Moreover, there are needs that have not yet been identified, or whose development leads to low or no expected profits. In this context, consumers might try to fulfill their desires by themselves. Users thus start their own problem solving by altering, changing, or adapting existing products or services, or even developing completely new ones. The result is a solution developed by a user to satisfy his own needs, usually referred to as a user innovation (von Hippel, 2007).

It has been documented that users, rather than manufacturers, are often the main source of innovation, constituting the dominant driving force in some industries (von Hippel, 1976, 1978; Thomke and von Hippel, 2002; von Hippel, 2007). von Hippel (1982) hypothesized that these disparities are caused by differences in the innovators' capacities

to appropriate the innovation benefit. In some cases, firms and manufacturers might also be user innovators, when they use a product or a service as an input in their production process. Users can be firms or individual consumers that benefit from using a given product or a service (von Hippel, 2007).

Quantification of user innovation

Since the 70's there has been a stream of research to identify, quantify, and study user innovation. Although initially user innovation was considered a rare event, as research progressed and more evidence is available, it became clear that it is in fact very common and covers a long list of areas, e.g.:

1. Scientific instruments, with 77% of user innovation, and 80% in the case of major innovations (von Hippel, 1976).
2. Semiconductor and electronic subassembly manufacturing equipment, with 63 % of user innovation (von Hippel, 1977).
3. Printed circuit CAD software, with 24% of user innovation (Urban and von Hippel, 1988).
4. Pipe hanger hardware design, with 36% of user innovation (Herstatt and von Hippel, 1992).
5. Library information systems, with 26% of user innovation (Morrison et al., 2000).
6. Mountain biking equipment, with 19% of user innovation (Lüthje et al., 2003).
7. Outdoor consumer products, with 10% of user innovation (Lüthje, 2004).
8. Commercial and retail banking services, with 85% of user innovation (Oliveira and von Hippel, 2011).

Determinants of user innovation

As the academic literature increasingly recognizes the phenomenon of user innovation, it becomes increasingly relevant to understand the characteristics of this sort of innovator as well as identify the determinants of such behavior so we can more easily identify users who have innovated or will do so.

Just like in the general topic of innovation, if users expect a large profit as a result of the development of a product, there will be a higher likelihood of innovation (Mansfield, 1968; Urban and von Hippel, 1988). Morrison et al. (2000) developed a study so as to

identify variables that help discriminate between innovators and non-innovators in a way that is easily captured via questionnaire. In this investigation seven variables were pointed out, four of which were considered to have a positive effect on the innovation by users. First, the Leading Edge Status, a notion further explained in Morrison et al. (2004), which is essentially the binary concept of "lead user" treated as a continuous variable through the aggregation of four measures. Just like Herstatt and von Hippel (1992) and Franke et al. (2006) have also pointed out, it has a positive impact on innovation. The other three positive variables are 1) the technical capability of the user, 2) the fact that they he/she is not able to find a suitably qualified third party that can be hired to carry out the innovation, and 3) the level of need, that is used as a proxy of the benefit expected from the innovation. Finally, if 1) the technical difficulties of innovating are perceived as more difficult, if 2) a producer, firm, supplier, etc. is receptive to perform the innovation itself, especially free of charge, and if 3) producers, firms, suppliers, etc. have a policy that discourages innovation by users, there will be a lower likelihood that innovation by users will take place. In terms of demographic variables, there is a higher probability of innovation in case the individual 1) is male, 2) holds an university degree, and 3) has a technical profession or education, e.g. science or engineering (von Hippel et al., 2011). In fact, if a person possesses these three characteristics the probability that he will innovate is up to 260% higher.

Diffusion of user innovation

One way to evaluate success and importance of an innovation is to consider the extent of its diffusion and adoption. In fact, as mentioned above, research suggests that users have originally developed a relevant portion of innovations in several fields that were diffused by becoming the basis for many new commercial products and services.

When a diffusion of an innovation is considered, there are several possible outcomes. One is that the innovator becomes an entrepreneur and commercializes the solution (Shah and Tripsas, 2007), or leaves the commercial part to another supplier or manufacturer. In many cases, e.g. scientific instrument innovation, the user is the primary actor in most of the innovation progress stages, leaving only the commercial diffusion to the manufacturer (von Hippel, 1976). Another outcome is that the innovator freely reveals the innovation to a community, as in a well-known case of open source software. Nowadays many users are able to get precisely what they need and properly develop their ideas, that later become a complement for manufacturer innovation (von Hippel, 2005). This shift in the role of consumers that were previously merely considered as "the market", led to a new innovation paradigm where consumers play an active role in the innovation process (Baldwin and von Hippel, 2011).

2.2.1 Patient innovation

A patient can be seen as a particular kind of user that expects significant benefits from the use of solutions that help him/her cope with his/her condition.

Although this research area is still recent and scarce, previous work has already showed how patients and their families have in fact innovated in the case of chronic diseases (Oliveira et al., 2011; Shcherbatiuk, 2012; Oliveira, 2012; Habicht et al., 2013). For example, close to 50% of the Treatments, Therapies & Medical Devices for Cystic Fibrosis were developed by patients. Furthermore, patients and their families increasingly sense a need to share their experiences and solutions with the remaining community. As the Information & Communication Technologies (ICT) spread, a number of online communities and blogs have flourished whereby patients are now sharing their experiences (Frydman, 2009). Interestingly, many of these, especially in the cases of Orphan Diseases, are transforming into Patient-driven research forums (Frydman, 2009; Wicks et al., 2010).

Potential of patient innovation for healthcare

As covered in the previous section, often driven by the desire to help the others patients share their experiences and solutions. This is well depicted in the words of Habicht et al. (2013),"In some cases the patients saved their own lives and end-up saving the lives of others". As the ICT become widely available, a number of online communities and blogs have flourished. Patients are now able to share their experiences and personal, health-related data[1]. Many of these communities, especially in the cases of orphan diseases, are transforming into actual patient-driven research communities. Albeit the value and knowledge created in such research forums, health suppliers and other stakeholders were for long hesitant to include and consider their ideas (Frydman, 2009; Wicks et al., 2010).

There are signals of a positive change, as healthcare experts are increasingly acknowledging the benefit and importance of the knowledge that patients and their families posses, encouraging patient collaborations (Bessant et al., 2012). In terms of healthcare stakeholders patients are the largest group, and they in fact possess expertize that not only is unique but also sometimes difficult to transfer. Moved by an urgent sense of need, and sometimes suffering from life-changing conditions, patients and their relative-caregivers become a promising source of ideas on how to improve processes and medical devices, or how to test new ways of treatment.

In their work Bessant et al. (2012) also refer to the benefits of an open innovation model in healthcare innovation. A new paradigm, where patients could more easily express their needs and collaborate from the beginning in the innovation process. By changing the

[1]This is the case of Patients Like Me (http://www.patientslikeme.com).

existing role of patients and medical practice, society's health could benefit more and faster, finally surpassing the current sense of healthcare innovation plateau. In fact, the online platform PatientsLikeMe[2] is a very promising example of an open innovation model that brings patients to the center of medical system (Kuenne et al., 2011).

2.3 Current healthcare model

The World Heath Organization defines health as "a state of complete physical, mental and social well-being and not merely the absence of disease or infirmity ... The enjoyment of the highest attainable standard of health is one of the fundamental rights of every human being without distinction of race, religion, political belief, economic or social condition." (World Health Organization, 2006).

In most developed countries, the current healthcare setting is marked by three main tendencies. Firstly the life expectancy at birth increase. It has risen more than 10 years in the past 40 years to average values of 80 years in the OECD countries, 80.8 years in Portugal, and 78.7 years in the US [3] (OECD, 2013b). The second is the ageing of the populations, as a result of diminishing birth rates and the referred increase in life expectancy. Many chronic conditions are age-related which means long-term heath care for more people and longer. Finally, and also related to the previous two, spending on health represents a very large and increasing share of both public an private expenditure. From 2000 to 2009 the average growth rate of health expenditure in OECD countries was circa 5%, which represents a growth substantially above the GDP growth. Although, this tendency has stagnated since 2010, health expenditure represents 9.5% of the GDP of OECD members, 10.2% in Portugal and 17.7% in the US, of which 72%, 66%, 48% respectively, are public expenses [4] (OECD, 2013a). The situation seems unsustainable.

Healthcare innovation

Under such scenario, the urge for innovation in processes that lead to higher levels of productivity, in new treatments, or a radical change in the whole healthcare system is pointed as the solution (Bessant et al., 2012). The current healthcare model however does not seem set to accept disruptive solutions that raise the quality of healthcare, but nonetheless threaten the status quo of key stakeholders (Christensen et al., 2000).

Currently, healthcare innovation has become an increasingly lengthy and costly process, particularly in the case of new drug development. DiMasi et al. (2003) estimated

[2] http://www.patientslikeme.com
[3] Values refer to the year of 2011.
[4] Values refer to the year of 2011.

that the average cost per new drug to the point of marketing approval is 802 million US$, which when compared to previous studies revealed an annual increase real rate of 7.4%. Regarding the length of the process, for standard cases[5], the average times for clinical phases is 6.5 years, and an additional 1.6 years in approval phase[6], which summed represent a total time of 8.1 years (Kaitin and DiMasi, 2010). Partly this is due to an enormous amount of bureaucracy involved, as a study by Dilts et al. (2009) has shown – in order to activate phase III trials there are at least 239 working steps, 52 major decision points, 20 processing loops and 11 stopping points.

Despite this disappointing scenario, the investment in R&D has increased in the past decades only the US Food and Drug Administration (FDA) approvals have not kept pace. As a response to the situation Paul et al. (2010) argues that without "a dramatic improvement in R&D productivity, the pharmaceutical industry cannot sustain sufficient innovation".

Involved players

Bessant et al. (2012) has identified and characterized the traditional five main players in the healthcare industry: Regulators, Providers, Payers, Suppliers and Patients. Although Regulators are the smallest group, they are the ones with greater influence as they set regulatory guidelines, include ministries of health as well as national or regional committees. Payers include private and public health insurance, as well as government agencies. Providers are all health professionals and medical experts providing care, and Suppliers are on the one hand the ones innovating by developing new treatments, e.g. scientific institutions as well as pharmaceutical and medical research companies, and on the other hand the ones reselling such as pharmacies. Finally, largest and least with least influence group, the Patients, which are the ones benefiting from care. This is represented in Figure 2.1.

The role of patients

In the previous context it becomes particularly interesting to understand to what extent patients prefer an active role in their own care. In fact, for long there has been significant research interest in understanding a patient's desire to be informed, as well as the extent to which patients wish to participate in medical treatment decision-making. These two dimensions consist of measures of patients' autonomy.

This has been a long debate, and authors have reached different conclusions in the case of decision-making. Regarding the desire for information authors seem to agree that indeed

[5]For priority cases, the total time is smaller by 1 year.
[6]Values relate to the period of 2005-2009.

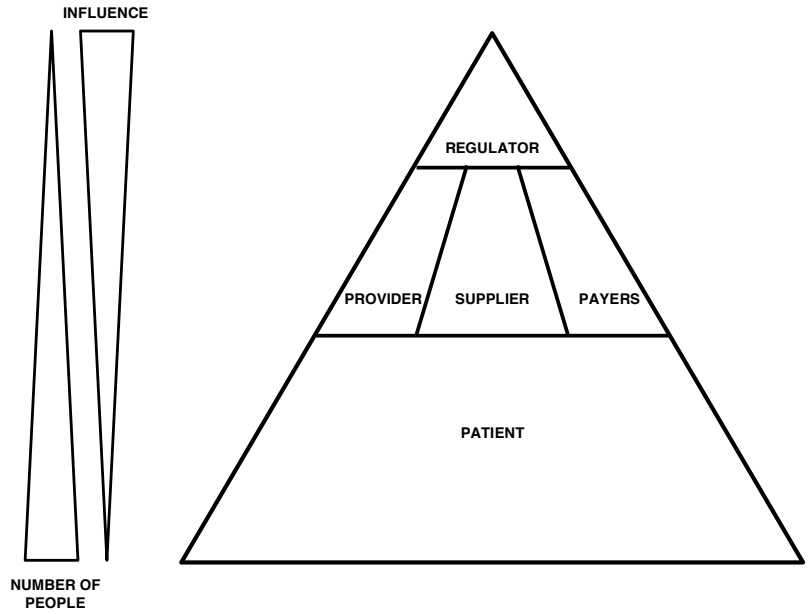

Figure 2.1: Five main players in the healthcare system.
Adapted from Bessant et al. (2012).

most patients prefer to be more informed about the diseases and treatment alternatives (Strull et al., 1984; Ende and Moskowitz, 1989; Deber et al., 1996; Levinson et al., 2005). Nonetheless, there are studies stating that patients indeed do not prefer to be involved in treatment decision-making Ende and Moskowitz (1989); Levinson et al. (2005), and other arguing that most patients do not wish to hand over the treatment decision-making to their physicians Strull et al. (1984); Deber et al. (1996). Moreover, patients' desire for autonomy in decision making is expected to increase farther 1) in the case of a life-threatening illness (Degner and Sloan, 1992), 2) in case there is more than one treatment alternative and 3) for younger patients (Guadagnoli and Ward, 1998). A more recent study by Levinson et al. (2005) found that 96% of patients preferred to be offered choices and to be asked their opinions, 52% do not wish to make final decisions and 44% rely only on information provided by their physicians. Factors increasing decision-making aspirations include 1) being female, 2) being more educated, 3) being healthier, and 4) being older up to 45 years. Factors decreasing decision-making desires include 1) being African-American and Hispanic, and 2) being older than 45 years. As most of complementary and alternative medicine (CAM) therapies are not accepted nor recommended by conventional medicine, much use of its use might represent clear examples of patient's autonomy in treatment decision-making. More details on CAM are given in Appendix A.

Finally, there is an ongoing debate on the notion of "patient empowerment" in the

health system. This idea includes topics as patient self-management, incentivizing patients to reach more information with their doctors, and encouraging the involvement of patients in treatment decision-making. Yet, there are still underlying assumptions, such as the notion that "health care providers support patient efforts to gain control", that require further examination (Harris and Veinot, 2004).

Chapter 3

Methodology

In the methodology section we will initially relate our research problem and questions to the approach we followed by determining our set of hypotheses (3.1). Hereafter we will make a thorough description of the collected data (3.2) as well as the methods chosen to test the hypotheses, and draw conclusions from the results (3.3).

3.1 Set of Hypotheses

The aims of our research are quantify innovation by patients, to identify its drivers and evaluate its importance. In order to address this problem statement we defined three research questions (RQ) and a hypothesis, whenever it is possible, for each of one of them. In this section, we will present the hypotheses and their rational.

RQ1: To what extent are patients and their families innovating?

Previous research (Oliveira et al., 2011; Shcherbatiuk, 2012; Oliveira, 2012; Habicht et al., 2013) already showed how patients and their families have in fact innovated and developed a series of non-drug medical solutions with the intention of using them. The case of rare diseases is particularly interesting for application of user innovation theory, as the market is underserved and characterized by a relatively large group of unsatisfied users (patients) (Griggs et al., 2009), with expectedly amplified needs. Accordingly, the hypothesis we will explore is:

H1: There is considerable prevalence of patient innovation among patients of rare diseases.

RQ2: What is the impact of patient innovation?

For a patient innovation to be meaningful it has to either ameliorate the health-status, or come as a solution to a problem. In other words, it should improve one's life quality (Nussbaum et al., 1993). For that reason we will measure importance through the improvement in life quality, thus hypothesizing:

H2: Patient innovation has a positive effect on patients' life quality.

RQ3: What factors contribute or withhold patient innovation?

Many antecedents of user innovation, demographic and non-demographic, have already been suggested by Morrison et al. (2000) and von Hippel et al. (2011). In this study we perform an exploratory analysis to verify to what extent some of these will hold in the case of patient innovation, as well as test new variables including disease and health-related ones. In table 3.1 of section 3.3.2 we will present all the tested variables and their hypothesized effect.

3.2 Data

Due to the specificity of our research problem we needed to collect a primary source of data for the purpose of our exploratory research. The data used for this thesis are collected within an international initiative for quantifying patient innovation, and we used the data collected in Portugal. We performed telephone survey interviews to 496 patients (202, i.e. 41%) and caregivers (293, i.e. 59%)[1] of rare diseases. We will now explain and describe 1) the survey design, 2) the data collection process, and innovation 3) validation and 4) coding.

3.2.1 Survey design

The survey development and data collection was conducted in the context of a project funded by the Peter Pribilla Foundation (Germany). Therefore the survey utilized builds upon an international tested and validated online survey for visualizing patient innovation. The original survey has been expanded to fit telephone interviews to patients of rare diseases and their caregivers in Portugal. Although only part of them will be used for the purpose of our research, the final survey has 73 questions in total. The survey has five different sections, each with a set of questions that aim at a specific objective. These five sections are:

[1]The missing case is an interviewee that is both a patient and a caregiver. For the purpose of our analysis this case will be treated as a patient.

1. **General questions about the disease and interviewee:** The aim of the questions in this section is to identify the type[2] of interviewee, as well as the disease, and the implications at all levels (degree of limitations, satisfaction with the existing treatment, and expenses) that the disease has in the interviewees' life.

2. **Questions about the innovation:** The first question of this section will try to identify if the interviewee has somehow developed a patient innovation. This question is asked indirectly as "Have you ever developed, adapted, or used in a way that is different from its original use, 1) a medical equipment, 2) a treatment, or therapy, or 3) a behavior, or habit, or strategy to better cope with the disease?", and an example is given so as to enable an easier identification of a possible solution. In case there is evidence of one or more innovations the person will be asked further questions regarding these solutions, otherwise, the interviewee will pass directly to the subsequent set of questions.

3. **Questions for non-innovators:** This set of questions is directed to interviewees that do not provide a possible patient innovation. These questions will cover the main reasons for not innovating as well as difficulties expected should they attempt to innovate.

4. **Demographic questions:** General demographic questions, including education and field of study, economic and marital status, family size, and age.

5. **ICT-related questions:** The aim of these questions is to measure the degree of openness to ICT. Interviewees are asked about their access to Internet and use of social networks (Facebook or other disease-related), as well as if they would be willing to join and share innovations in an online platform.

All the questions that were used for the purpose of our research are presented in detail in Appendix B.

3.2.2 Data collection

The data collection process was performed in a partnership with Raríssimas – Associação Nacional de Doenças Mentais e Raras[3], a Portuguese association for both mental and rare diseases. Information about potential contacts was obtained from their database and a random sample of patients and caregivers was collected. All the interviews were carried out by four employees of Raríssimas. These experts' professional activity involves talking to patients and caregivers of rare diseases on a daily basis as they are in charge of the

[2]Caregiver or patient.
[3]http://www.rarissimas.pt/

telephone help-line for patients. They were therefore chosen to perform the interviews so as to extract the highest possible level information, and maximize the response rate[4]. Although there was a script with specific questions to follow, interviews were made in a conversational style and thus took on average approximately 30 minutes.

3.2.3 Innovation validation

As von Hippel et al. (2011) asserted, it is essential to "eliminate *false positives* – claimed innovations which were in fact not innovations (such as, *I bought a piece of IKEA furniture and put it together myself*)". For this reason we had to analyze all the collected innovations individually and exclude false ones on the following grounds[5]:

1. **Mistake:** Innovations that should have been validated directly by the interviewer as the patient either 1) said the innovation produced no result, or 2) said the solution was recommended by the doctor.

2. **Common sense:** Obvious solutions fall on this category, that is perhaps best illustrated by examples: "The medication dried the skin, so I used a good moisturizer", or, "I removed slippery carpets to avoid falling".

3. **Not innovative:** On the one hand, we have obvious cases of solutions that already exist in the market, such as the use of a lace wig or a pillbox. Additionally, it excludes cases of a known solution that produced usual benefits. An example would be a solution as "practice of extracurricular activities to improve the general and physical wellbeing and avoid deprecation", or "physiotherapy to improve mobility". As opposed to these situations, a known solution that is used with a different purpose and produces new results, such as the practice of martial arts, which was discouraged by the doctor, leading to internal and external bleeding stopping, is considered innovative.

3.2.4 Innovation coding

When it comes to user innovation in the field of healthcare the user not only has to overcome the usual barrier of the process of innovation but also the psychological barrier of being autonomous in terms of treatment decision-making due to the responsibility of its inherent risks. Thus, we consider that to become a patient innovator, the person first has to be active in the sense of assuming the responsibility of undertaking a treatment beyond doctor recommendations. In this sense we developed a coding decision-model that

[4]There were very few cases of interviewees rejecting to answer the questionnaire.
[5]The examples that will be provided are real.

is illustrated in Figure 3.1. The objective is to sort the solutions, in the following four categories:

1. **Passive:** Solutions that are used as common treatment. These include non-innovators, and solutions that were referred as being prescribed by the doctor.
2. **Active:** Existing solutions that are not always used as common treatment. An example would be a drug that is only used in some countries. Additionally, it includes existing solutions that are not recommended in common treatment, i.e. those CAM treatments that are not yet accepted by conventional medicine.
3. **Patient Innovator:** Solutions that are completely new-to-the-market.

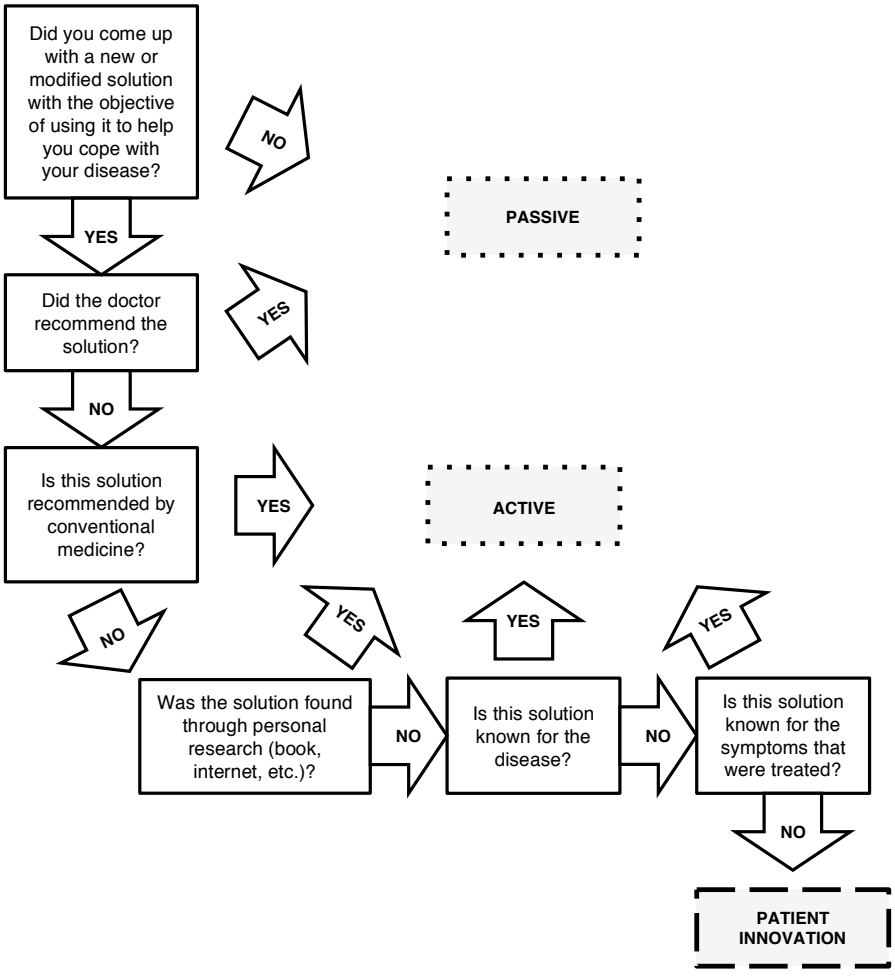

Figure 3.1: Innovation coding model.

The rational behind the model is that there are two main types of patients and caregivers, those that are passive and do merely what the doctor advises, and those that are

active. Active patients are to some extent autonomous in their treatment decision-making, i.e. they seek alternatives and try other solutions by themselves. In this context, patient innovations are innovative solutions that active patients or caregivers developed. This is depicted in Figure 3.2.

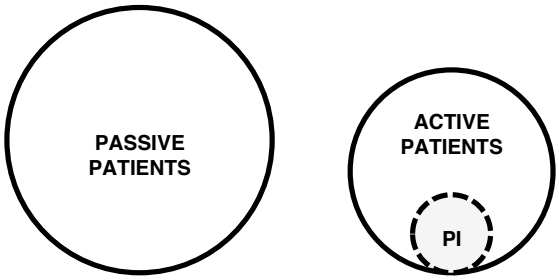

Figure 3.2: Patient innovation represented as an active-innovative solution.

For the purpose of our analysis we are interested in studying the patient innovation phenomenon, yet it is relevant to also analyze characteristics of active patients, as these might be in a stage that anticipates becoming a patient innovator.

3.3 Methods

For the purpose of our data analysis we will use two methods: descriptive statistics (3.3.2), specifically when addressing RQ 1 and RQ 2 and as a means to provide some information regarding the collected data, and a multiple logistic regression for RQ 3, in order to identify relevant predictors of patient innovation and active patients.

3.3.1 Descriptive Analysis

As was covered in the previous section, we quantified the number of patient innovations in the sample, which resulted in two groups, patient innovators and non-patient innovators. To test hypothesis 1 (*"There is considerable prevalence of patient innovation among patients of rare diseases."*) we will quantify the proportion of patient innovation. Although the populations have different characteristics, as we know a priori that patients have a high need in a specific disease, we will verify if our results are in line with those of other user innovation research findings (see section 2.2.

Hypothesis 2 (*"Patient innovation will have a positive effect on patients' life quality."*) only concerns the patient innovation cases. In the survey, these interviewees are asked to rate life-quality on a 7-point Likert scale ("No life quality" to "Excellent life quality")[6] before and after the innovation. We will verify to what extent there is a perceived

[6]We opted for a large scale as to measure improvement a high level of precision is required.

improvement in life-quality due to the innovation.

3.3.2 Regression Analysis

Through RQ 3 (*"What factors contribute or withhold patient innovation"*) we aim at identifying statistically relevant predictor variables of patient innovation, consequently we performed a regression analysis. The dependent variable is dichotomous (being a patient innovator or not), and therefore we used a logistic regression. The most important outcome measure to interpret the logistic regression results is the odds ratio (OR), which is an indicator of the change in odds of the independent variable occurring resulting from a unit change in the predictor variable (Field, 2009). Thus for the purpose of our analysis we will only consider predictor variables, those with $P < 0.05$, and a 95% confidence interval (CI) > 1 for the OR. We will now describe in detail the model building process.

Model building

Our main dependent variable is the dichotomy of being or not a patient innovator. Nonetheless as mentioned previously, we will also perform the analysis for the case of a patient being active or passive. Concerning the independent variables, according to Hult et al. (2004) the best way to address effects of innovativeness and its antecedents is not through an unrealistic analysis of bivariate relationships, but rather through a more integrated approach, thus we opted for a multivariate model. We entered the demographic predictor variables in a first block and in a second block we included the remaining variables together, as there was no apparent rational to separate them in different blocks. In Table 3.1 we summarize the independent variables of each block, as well as the hypothesized effect on patient innovativeness. In Appendix C we provide further information regarding the measurement of each variable.

An additional analysis we will perform is the intercorrelation between the tested variables, as multicollinearity can be substantially problematic (Field, 2009). Although there is no definite value above which a correlation[7] is considered significant or too high, we will consider the rule-of-thumb cut values defended by Cohen (1988): $r = .10$ (small effect), $r = .30$ (medium effect) and $r = .50$ (large effect).

[7] We will use the Pearson product-moment correlation coefficient r.

VARIABLE	HYPOTHESIZED EFFECT
Block 1: Demographic variables.	
Age	***
Gender	Male are more likely to innovate (Von Hippel et al., 2011).
Education	Holding a university degree has a positive impact (Von Hippel et al., 2011).
Marital Status	***
Professional Situation	***
Block 2: Non-demographic variables.	
Number of years with the disease	Positive effect, as the knowledge and experience with the disease is higher.
Type of disease: ICD-10 code	***
Relationship with the patient	Patients, as well as parents, due to the nature of the relationship, are more likely to innovate.
Limitations in daily life	Positive effect, as the expected benefits of innovating will be higher.
Dissatisfaction with available treatment	Positive effect, as the motivation to innovate is higher.
Expenses awareness	Positive effect because a person that is aware of the expenses with the disease expresses concern and control about the disease and impacts it has in daily life.
ICT readiness	Positive effect, due to the inherent acquired skills and knowledge.

*** Cases for which a priori we could not develop a theory

Table 3.1: Independent variables.

Chapter 4

Results

We will present the results of our exploratory research following the structure of our research questions and proposed hypotheses. Thus we will first cover the encountered prevalence of patient innovation (4.1), followed by the identification of its antecedents (4.2) and finally the impact it has on patients' quality of life (4.3).

4.1 Prevalence of patient innovation

From the 496 interviewees inquired, 255 (51%) reported they had developed at least one possible solution. More specifically, 220 (44%) reported one solution, 33 (7%) reported two solutions and 2 interviewees (0.4%) reported three solutions, thus totalizing 292 solutions developed by 255 interviewees. Moreover, the 292 possible solutions were sorted into the following types: 1) a medical equipment, 2) a treatment, or therapy, or 3) a behavior, or habit, or strategy. The rarest type of solution was medical equipment (10%, 30 cases), and the most common (50%, 146 cases) were treatments or therapies. In Table 4.1 we summarize the information regarding all the solutions that were collected.

SOLUTIONS PER INTERVIEWEE				
0 solu.	1 solu.	2 solu.	3 solu.	**total**
241	220	33	2	**496**

TYPE OF SOLUTIONS	
Medical Equipment	30
Treatment or therapy	146
Behavior, habit or strategy	116
total	**292**

Table 4.1: Solutions before the validation. (N=496)

After the proposed solutions were validated, a total of 223 (76%) false positives were

excluded due mainly to not being innovative (146 cases). Thus, only 69 solutions are patient innovations. Although only 24% were considered patient innovations, we nonetheless recognized that 179[1] (61%) of the provided solutions are examples of active patients' actions, i.e. cases where patients decided to pursuit a given treatment beyond, and in many cases against, the instructions given by the doctor. In this sense, after the validation was performed, we encountered a total of 159 (32%) active patients in our sample, of which 62 (13%) are patient innovators as demonstrated in Figure 4.1. These findings allow us to conclude that our first hypothesis, *"There is considerable prevalence of patient innovation among patients of rare diseases."*, is valid, as the proportion of patient innovation is in line with those of other user innovation research findings (Lüthje et al., 2003; Lüthje, 2004, among others).

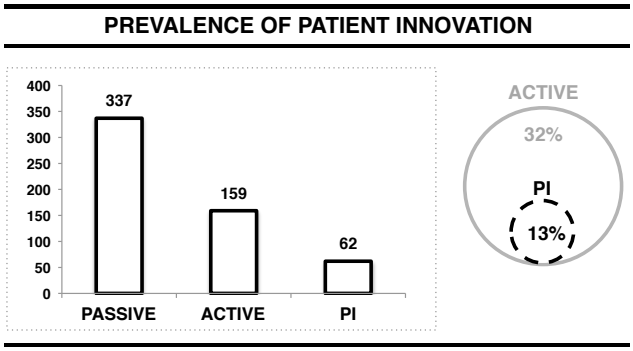

Figure 4.1: Prevalence of patient innovation. (N=496)

4.2 Impact of patient innovation

Interviewers asked all the patients who reported an innovation to rate on a 7-point scale (*1 - non-existing* to *7 - excellent*), the quality of life of the patient before and after their innovation took place. Figure 4.2 depicts the answers to these two questions regarding each patient innovation. As is evident in the histogram, due to the shift to the left of the "after" curve, there is a very strong improvement in the quality of life after the innovations. Equally relevant is the fact that after the innovation the options *1 - non-existing quality of life* and *2 - very little quality of life* were no longer chosen.

Furthermore, the average rate given to the quality of life of the patient before the innovation was 2.92, i.e. close to *3 - little quality of life*. With a 2.43 (an extra 83%) point improvement the average quality of life reaches the value of 5.35 after the innovation, staying between *5 - considerable* and *6 - high*. In fact, as is depicted in Figure 4.3 there

[1]Including the patient innovations.

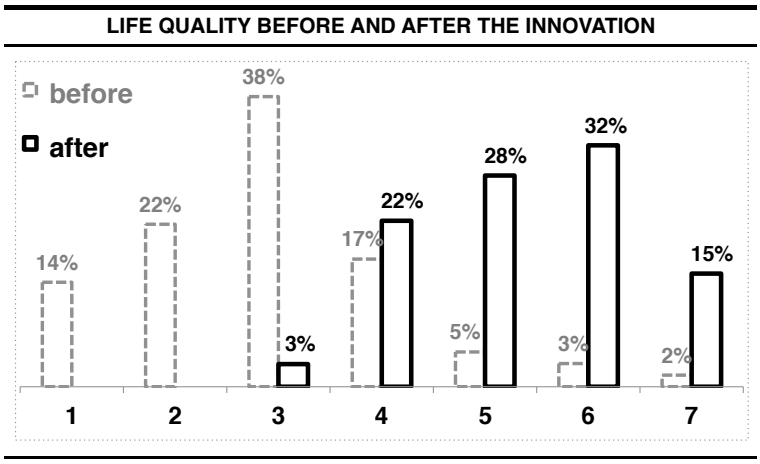

Figure 4.2: Patients' life quality before and after the innovation. (N=65)

are no cases of a decrease in the quality of life, and only seven innovations (11%) resulted in the maintenance of the same level of life quality. Therefore, we find support for our second hypothesis (*"Patient innovation will have a positive effect on patients' life quality"*), as the innovations proved to have a significant and positive impact.

IMPROVEMENT IN QUALITY OF LIFE

	Total	Relative percentage
Average life quality before the innovation (a)	2.92	55% (a/b)
Average life quality after the innovation (b)	5.35	183% (b/a)
Total improvement in quality of life (b-a)	**2.43**	***

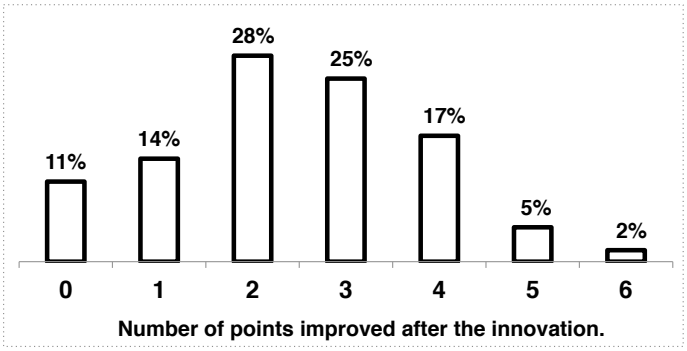

Figure 4.3: Post-innovation improvement in the quality of life of the patients. (N=65)

4.3 Antecedents of patient innovation

The results of the survey regarding the selected variables for the model are presented in Appendix D. In Table D.1 we present the demographic variables, and in Table D.2 the

non-demographic variables.

Concerning the intercorrelations between all the variables included and being a patient innovator (PI), none are highly correlated ($r \geq .50$). From all the variables, the most correlated pair is professional situation and age ($r = .42$ with $P < .01$). Regarding the variables included in the model there are only two pairs of correlated variables, with only a small effect. The pairs are holding a university degree and professional situation ($r = -0.27$, with $P < .01$), and ICT readiness and professional situation ($r = 0.26$, with $P < .01$). Table 4.2 presents the intercorrelations between all the hypothesized variables and patient innovativeness.

INTERCORRELATIONS BETWEEN HYPOTHESIZED VARIABLES

	1.	2.	3.	4.	5.	6.	7.	8.	9.	10.	11.	12.	13.
1. Patient Innovator	***												
2. Age	-0.05	***											
3. Gender	-0.02	0.08	***										
4. Holding a university degree	0.07	-0.14**	0.04	***									
5. Marital status	-0.03	0.02	0.04	-0.02	***								
6. Professional situation	0.03	0.42**	0.09*	-0.27**	-0.06	***							
7. Years with the disease	-0.01	0.16**	-0.09*	-0.03	0.06	0.15**	***						
8. ICD10 disease code	-0.10*	0.13**	0.07	-0.01	0.03	0.07	-0.06	***					
9. Relationship with the patient	-0.04	0.00	0.04	0.12**	0.06	-0.03	-0.02	-0.11*	***				
10. Limitations in daily life	0.08	0.07	-0.01	-0.12**	0.00	0.19**	0.08	-0.03	0.08	***			
11. Dissatisfaction with treatment	0.11*	-0.10*	-0.05	0.05	0.08	-0.08	0.04	-0.15**	0.01	0.27**	***		
12. Expenses awareness	0.20**	-0.03	-0.03	0.03	-0.04	-0.05	-0.04	-0.15**	-0.15**	0.14**	0.16**	***	
13. ICT readiness	0.13**	-0.37**	-0.04	0.26**	-0.04	-0.36**	-0.08	-0.20**	0.00	-0.11*	0.11*	0.03	***

* P < 0.05

** P < 0.01

Table 4.2: Intercorrelations of hypothesized predictor variables.

There were both demographic and non-demographic variables predicting (with a $P < 0.05$) a person being a patient innovator in the multiple logistic regression. The demographic variables were: (1) holding a university degree, (2) being single, which has a negative impact, (3) being unemployed, and (4) looking after home as a profession. Regarding non-demographic variables these were: (1) expenses awareness, and (2) ICT readiness. Due to missing information in some data points (i.e., the answers "Prefer not to answer" and "Non applicable") only 437, of the 496, were included in the regression. Table 4.3 contains the odds ratio significance and 95% confidence intervals from all the variables that arose as significant predictors.

The outcome of fitting an identical multiple logistic regression model to the active patient dependent variable results in quite similar conclusions. As is represented in Table 4.4, from the six identified predictors of patient innovators, four are also predictors of active patients or caregivers: (1) holding a university degree, (2) looking after home as a profession, (3) expenses awareness, and (4) ICT readiness.

SIGNIFICANT PREDICTORS OF PATIENT INNOVATION			
	SIG.	OR	OR 95% C.I.
Holding a university degree	.036	1.88	(1.04 - 3.40)
Marital status: single	.041	0.38	(0.15 - 0.96)
Professional situation: unemployed	.047	2.12	(1.01 - 4.47)
Professional situation: looking after home	.013	3.78	(1.33 - 10.7)
Expenses awareness	.003	2.65	(1.40 - 4.99)
ICT readiness	.021	1.73	(1.09 - 2.77)

Table 4.3: Significant predictors of patient innovation in the multiple logistic regression.

SIGNIFICANT PREDICTORS OF ACTIVE PATIENTS			
	SIG.	OR	OR 95% C.I.
Holding a university degree	.000	2.19	(1.44 - 3.33)
Professional situation: looking after home	.034	2.48	(1.02 - 5.73)
Expenses awareness	.007	1.86	(1.18 - 2.92)
ICT readiness	.002	1.52	(1.16 - 2.00)

Table 4.4: Significant predictors of active patients in the multiple logistic regression.

As we show in Table 4.5, two of the variables from which no effect was foreseen proved to have some impact in the likelihood of a person being a patient innovator, these are (1) marital status: single; and (2) professional situation: unemployed and looking after home. Moreover, not all the individual hypothesized effects of the variables were confirmed in the regression. Five variables that were expected to have influence, in fact do not: (1) age, (2) the number of years with the disease, (3) the relationship with the patient, (4) the perceived limitations in daily life, and (5) the dissatisfaction with the available treatment. Yet, there are possible interpretations for all the revealed effects.

Let us first start with the demographic variables. In the case of gender, the usual higher propensity of males to be user innovators is not verified. A possible explanation is that when it comes to health issues, the need and perceived benefits of innovating are equally distributed among genders. In fact, as demonstrated in Table 4.2 gender and PI are not correlated ($r = .02$). Education, i.e. holding a university degree, as expected is an antecedent of PI. This variable reveals that not only the subject is more likely to have the required technical skills to innovate, but also that possesses a higher level of knowledge. Being single, when compared to being married, was shown to have a negative impact. This can possibly be explained by the fact that the person in question will have less support as consequently feel more apprehensive about innovating. Finally, when compared to employed, unemployed and looking after home individuals, are also more likely be a PI. These cases have both one possible thing in common: more disposable time. Moreover, these findings might also suggest that these cases include to a great extent either people

VARIABLE	HYPOTHESIZED EFFECT	ACTUAL EFFECT	STATUS
Block 1: Demographic variables.			
Age	***	No effect.	***
Gender	Positive effect for male.	No effect.	✗
Education	Positive effect.	Positive effect.	✓
Marital Status	***	Negative effect for: single.	***
Professional Situation	***	Positive effect for: unemployed and looking after home.	***
Block 2: Non-demographic variables.			
Number of years with the disease	Positive effect.	No effect.	✗
Type of disease: ICD-10 code	***	No effect.	***
Relationship with the patient	Positive effect for patients and parents.	No effect.	✗
Limitations in daily life	Positive effect.	No effect.	✗
Dissatisfaction with available treatment	Positive effect.	No effect.	✗
Expenses awareness	Positive effect.	Positive effect.	✓
ICT readiness	Positive effect.	Positive effect.	✓

*** Cases for which a priori we could not develop a theory

Table 4.5: Actual effect of the independent variables.

whose lives are highly conditioned by the disease or that, especially in the case of caregivers, devote their life to the disease.

Considering the non-demographic variables, there at least two possible explanations for the number of years with disease have proven to have no effect on PI. Although a higher level of expertise would be expectable, as time passes, there might also be the tendency to (1) accept the disease and lose hope and (2) be more incapacitated. A higher level of perceived limitations in daily life also revealed not to influence PI despite a higher benefit of innovating was expected. An explanation for such an outcome may be the variable itself, as a person whose life is more limited will less likely be prone to innovate. As for the lack of relationship of a higher level of dissatisfaction with the available treatment and the likelihood of innovation, possible explanations include (1) individuals that complain the most are not necessarily those that will more likely take action, and (2) treatments for rare diseases are very scarce, therefore most of the population is naturally dissatisfied, and the effect is mitigated.

Nevertheless, the outcomes of our analysis allow us to conclude that indeed the antecedents of patient innovators include both demographic – (1) holding a university degree, (2) being single, which has a negative impact, (3) being unemployed, and (4) looking after home as a profession – and non-demographic factors – (1) expenses awareness, and (2) ICT readiness).

Chapter 5

Conclusions

In this chapter we will first provide a detailed analysis of the implications of our findings (5.1). Than we will identify the existing limitations of our study (5.2) that, as will be explained, will in some cases consist of opportunities for future research, a topic that will be covered in section 5.3.

5.1 Implications of our research

As was presented, we observed a significant existence (32%) of patients and caregivers of rare diseases that seek new solutions and start treatments beyond the recommendations of the doctor, showing that to some extent they are autonomous in their treatment decision-making. Among this group, some (13%) take the additional responsibility to go even further and try completely new-to-the-market solutions, that they themselves developed and that until that moment had not been tested. We refer to this group as patient innovators, and the identification of its members is of the utmost importance for society (Habicht et al., 2013). If the healthcare system is able to identify these individuals and include them in a new and more collaborative healthcare research model, their innovations will have the chance to reach a broader audience. In this way other patients facing the same conditions will have the chance to choose between a wider set of medical equipment, treatments and other solutions to better cope with their symptoms. Moreover, as was suggested by our research, these patients might possibly increase their quality of life in a very substantial way.

In this sense, the first conclusion to take from this dissertation is that there should be an increased effort to identify possible patient innovators. As we can see in Table 5.1 in the presence of all the identified predictor variables of our study, the likelihood that a patient or caregiver will innovate is 320% higher than the likelihood of an average patient or caregiver of doing so. These and other findings can be very helpful to address the issue

PROPORTION OF PI IN THE PRESENCE OF PREDICTOR VARIABLES		
	percentage of PI	**increase**
Whole sample	**12.5%**	***
Holding a university degree	15.3%	1.2
Marital status: single	7.4%	0.6
Professional situation: unemployed	16.7%	1.3
Professional situation: looking after home	23.3%	1.9
Expenses awareness	21.4%	1.7
ICT readiness	15.6%	1.3
Presence of all the variables	**40%**	**3.2**

Table 5.1: Percentage of patient innovators in the presence of the predictor variables.

of making the voices of PI's being heard.

The clear positive impact that the innovations produced in the lives of the patients is more than a sufficient reason to acknowledge the great potential that these innovations might have in the limited lives of yet so many underserved patients. In their constant debate for reforms in the present health care system, policymakers, regulators and health care professionals, should now have added reasons to at least consider a way in which these innovations can be incorporated, tested and approved in the system.

In fact, another interesting aspect of our findings is the significant existence of active patients, individuals that indeed are willing to risk more, maybe because in some cases they do not have that much to lose. This suggests that our current healthcare system is not only perhaps overprotective but paternalistic too. Paternalistic in the sense that the pyramid of players in the healthcare system (Figure 2.1) gives no power at all to their largest group: patients. Maybe the active individuals among that group could play important roles, even if not that of innovating. They seem to be very willing to go beyond mainstream medicine.

Building upon the three-phase new innovation paradigm developed by von Hippel et al. (2011), we can conceive one interesting scenario where active patients somehow test and evaluate (corresponding to phase 2 in Figure 5.1) the potential of patient innovations. For instance, pharmaceuticals and other producers would then be able to produce these innovations as soon as their potential became clearer. This is one clear situation where recognizing active patients through the identification of their distinctive characteristics can be so valuable.

Finally and in sum, there are numerous cases of patient innovation that are important for society, as they were perceived to produce a positive impact on the quality of life of patients. In this sense it may be beneficial to explore further how to identify, validate, improve and diffuse these innovations and exploit this important innovative force of patients, so others can take advantage of their benefits in the future.

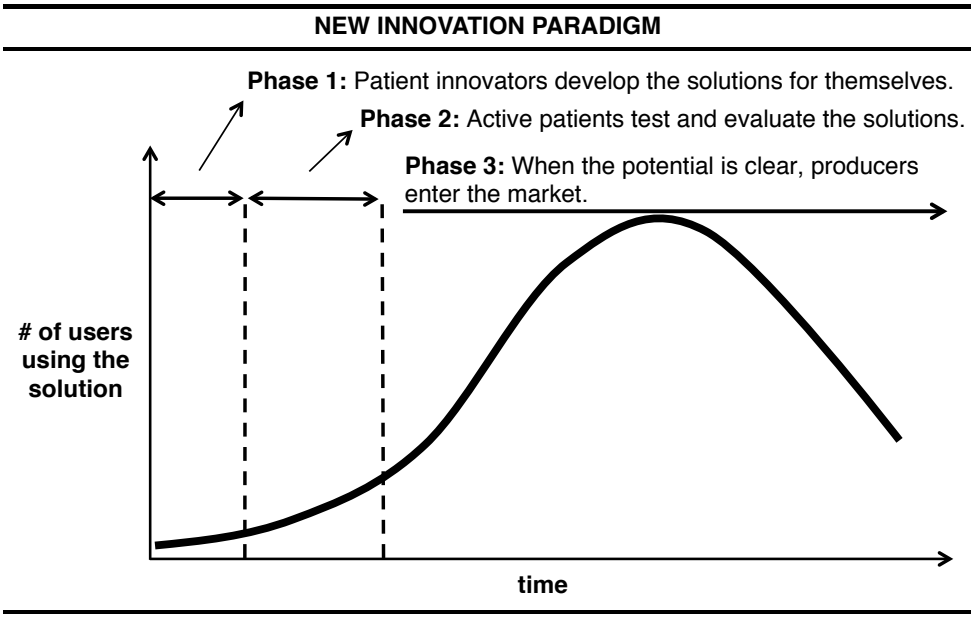

Figure 5.1: Three-phase new innovation paradigm.
Adapted from von Hippel et al. (2011).

5.2 Limitations

There are some limitations in our work with implications that should be taken into account when examining our results and conclusions. These limitations include:

- Although we consider our survey data very rich due to its comprehensiveness, the sample size might, in some analysis, be small as there are many data combinations with few occurrences. In this sense, our study would benefit from a bigger sample.
- The sample might not be, in some cases, representative of the population, as the random sample was taken from the database of Raríssimas' phone line for patient-support. This data-base consists of individuals, both patients and caregivers, who contacted Raríssimas.
- The validation of the patient innovations was based on internet research and completed internally, nonetheless a validation made by an expert panel, more specifically by doctors, is a lacking requisite..
- The fact that we are using self-reported data which might be subject to distortion and inaccuracy.
- The measurement of patients' quality of life before and after the innovation is not objectively measured and validated. Hence, this assessment may be biased due to

consisting of a self-analysis of one's innovation. This is a serious limitation that can compromise the validity of the results, as self-reported measures are very susceptible to biases (Hoskin, 2012).

- There is a significant amount of variance in the independent variables that is not explain by the explanatory variables. Nonetheless this is common in any study attempting to explain a complex human behavior.

- Just as in any regression analysis, we are not able to uncover sure causal relationships between the variables.

Despite these limitations, we nonetheless consider that our research made some contribution to the study of patient innovation.

5.3 Future research

Many of the identified limitations can be seen as future research opportunities, as our study would undoubtedly be more conclusive if those limitations were overcome.

We studied rare diseases in particular because we expected to find there a more significant existence of PI among these patients. Therefore it would be interesting to analyze if the results are maintained beyond the borders of orphan diseases. Similarly, we made no distinction between patient innovators and caregiver innovators, and a cross-sectional analysis could also be of interest in this point.

Regarding the study of the antecedents of patient innovators, there are a series of unaccounted variables in our study, such as psychological traits, environmental conditions, openness to experimentation, or even risk preferences that may contribute to the predisposition of patients and caregivers to innovate. Future research should address these variables.

Finally it is of the utmost importance to identify ways in which the safety and validity of the innovations can be tested, thus this sort of models should be examined in future studies.

Bibliography

Agarwal, R. and Prasad, J. (1997). The role of innovation characteristics and perceived voluntariness in the acceptance of information technologies. *Decision Sciences*, 28(3):557–582.

Baldwin, C. and von Hippel, E. (2011). Modeling a paradigm shift: from producer innovation to user and open collaborative innovation. *Organization Science*, 22(6):1399–1417.

Baregheh, A., Rowley, J., and Sambrook, S. (2009). Towards a multidisciplinary definition of innovation. *Management Decision*, 47(8):1323–1339.

Barnes, P. M., Powell-Griner, E., McFann, K., and Nahin, R. L. (2004). Complementary and alternative medicine use among adults: United States, 2002. In *Seminars in Integrative Medicine*, volume 2, pages 54–71. Elsevier.

Bessant, J., Künne, C., and Möslein, K. (2012). Opening up healthcare innovation: Innovation solutions for a 21st century healthcare system. Technical report, Advanced Institute of Management Research.

Chan, E. (2008). Quality of efficacy research in complementary and alternative medicine. *JAMA: The Journal of the American Medical Association*, 299(22):2685–2686.

Chesbrough, H. W. (2003). *Open innovation: The new imperative for creating and profiting from technology*. Harvard Business Press.

Chesbrough, H. W. (2006). The era of open innovation. In *Managing innovation and change*, pages 34–41. Sage Publications, 3rd edition.

Christensen, C. M., Bohmer, R., Kenagy, J., et al. (2000). Will disruptive innovations cure health care? *Harvard Business Review*, 78(5):102–112.

Christensen, C. M., Cook, S., and Hall, T. (2005). Marketing malpractice: the cause and the cure. *Harvard Business Review*, 83(12):74–83.

Cohen, J. (1988). *Statistical power analysis for the behavioural sciences*. New York: Academic Press.

Damanpour, F. (1991). Organizational innovation: A meta-analysis of effects of determinants and moderators. *Academy of Management Journal*, pages 555–590.

Deber, R. B., Kraetschmer, N., and Irvine, J. (1996). What role do patients wish to play in treatment decision making? *Archives of Internal Medicine*, 156(13):1414.

Degner, L. F. and Sloan, J. A. (1992). Decision making during serious illness: what role do patients really want to play? *Journal of Clinical Epidemiology*, 45(9):941–950.

Denning, P. and Dunham, R. (2010). *The Innovator's Way: Esssential Practices for Successful Innovation*. The MIT Press.

Denning, P. J. (2012). Innovating the future: From ideas to adoption. *The Futurist*, pages 41–45.

Dilts, D., Sandler, A., Cheng, S., Crites, J., Ferranti, L., Wu, A., Finnigan, S., Friedman, S., Mooney, M., and Abrams, J. (2009). Steps and time to process clinical trials at the cancer therapy evaluation program. *Journal of Clinical Oncology*, 27(11):1761–1766.

DiMasi, J., Hansen, R., Grabowski, H., et al. (2003). The price of innovation: new estimates of drug development costs. *Journal of Health Economics*, 22(2):151–186.

Dormann, J., Holliday, C., et al. (2002). *Innovation, technology, sustainability and society*. WBCSD.

Drucker, P. F. (1985). The discipline of innovation. *Harvard Business Review*, 63(3):67.

Eisenberg, D. M., Davis, R. B., Ettner, S. L., Appel, S., Wilkey, S., Van Rompay, M., and Kessler, R. C. (1998). Trends in alternative medicine use in the United States, 1990-1997. *JAMA: The Journal of the American Medical Association*, 280(18):1569–1575.

Ende, J. and Moskowitz, M. A. (1989). Measuring patients' desire for autonomy. *Journal of General Internal Medicine*, 4(1):23–30.

EURORDIS (2005). Rare diseases: understanding this public health priority.

Field, A. (2009). *Discovering statistics using SPSS*. Sage Publications.

Fontanarosa, P. B. and Lundberg, G. D. (1998). Alternative medicine meets science. *JAMA: The Journal of the American Medical Association*, 280(18):1618–1619.

Franke, N., von Hippel, E., and Schreier, M. (2006). Finding commercially attractive user innovations: A test of lead-user theory. *Journal of Product Innovation Management*, 23(4):301–315.

Frankelius, P. (2009). Questioning two myths in innovation literature. *The Journal of High Technology Management Research*, 20(1):40–51.

Frydman, G. (2009). Patient-driven research: rich opportunities and real risks. *Journal of Participatory Medicine*.

Griggs, R. C., Batshaw, M., Dunkle, M., Gopal-Srivastava, R., Kaye, E., Krischer, J., Nguyen, T., Paulus, K., and Merkel, P. A. (2009). Clinical research for rare disease: opportunities, challenges, and solutions. *Molecular Genetics and Metabolism*, 96(1):20–26.

Guadagnoli, E. and Ward, P. (1998). Patient participation in decision-making. *Social Science & Medicine*, 47(3):329–339.

Habicht, H., Oliveira, P., and Shcherbatiuk, V. (2013). User innovators: When patients set out to help themselves and end up helping many. *Die Unternehmung*.

Harris, R. and Veinot, T. (2004). The empowerment model and using e-health to distribute information. *Vancouver, BC: Action for Health, Simon Fraser University and the Vancouver Coastal Health Research Institute*.

Herstatt, C. and von Hippel, E. (1992). From experience: Developing new product concepts via the lead user method: A case study in a "low-tech" field. *Journal of Product Innovation Management*, 9(3):213–221.

Hoskin, R. (2012). The dangers of self-report. Unpublished blog post http://www.sciencebrainwaves.com/uncategorized/the-dangers-of-self-report, Brainwaves: science for all.

Hult, G. T. M., Hurley, R. F., and Knight, G. A. (2004). Innovativeness: its antecedents and impact on business performance. *Industrial Marketing Management*, 33(5):429–438.

Jaruzelski, B. and Dehoff, K. (2008). Beyond borders: the global innovation 1000. *Strategy+business*, 53(Winter):52–69.

Kaitin, K. and DiMasi, J. (2010). Pharmaceutical innovation in the 21st century: new drug approvals in the first decade, 2000–2009. *Clinical Pharmacology & Therapeutics*, 89(2):183–188.

Kalmanek, C. (2012). The essential elements of successful innovation. *ACM SIGCOMM Computer Communication Review*, 42(2):105–109.

Kandybin, A. (2009). Which innovation efforts will pay. *Sloan Management Review*, 51(1):53–60.

Kuenne, C., Rass, M., Adamcyzk, S., Bullinger, A., and Moeslein, K. M. (2011). Patients as innovators: An open innovation perspective on health 2.0. *Proceedings of MCPC*.

Länsisalmi, H., Kivimäki, M., Aalto, P., and Ruoranen, R. (2006). Innovation in healthcare: a systematic review of recent research. *Nursing Science Quarterly*, 19(1):66–72.

Levinson, W., Kao, A., Kuby, A., and Thisted, R. A. (2005). Not all patients want to participate in decision making. *Journal of General Internal Medicine*, 20(6):531–535.

Levitt, T. (2002). Creativity is not enough. *Harvard Business Review*, 80:137–144.

Lüthje, C. (2004). Characteristics of innovating users in a consumer goods field: An empirical study of sport-related product consumers. *Technovation*, 24(9):683–695.

Lüthje, C., Herstatt, C., and von Hippel, E. (2003). The dominant role of "local" information in user innovation: The case of mountain biking. Working paper 4377-02, MIT Sloan School of Management.

Mansfield, E. (1968). *Industrial research and technological innovation: An econometric analysis*. Norton New York.

Morrison, P., Roberts, J., and von Hippel, E. (2000). Determinants of user innovation and innovation sharing in a local market. *Management Science*, 46(12):1513–1527.

Morrison, P. D., Roberts, J. H., and Midgley, D. F. (2004). The nature of lead users and measurement of leading edge status. *Research Policy*, 33(2):351–362.

Nås, S. O. and Leppãlahti, A. (1997). Innovation, firm profitability and growth. STEP Report series 199701, The STEP Group, Studies in technology, innovation and economic policy.

Nussbaum, M. C., Sen, A. K., and Sugden, R. (1993). *The quality of life*. Clarendon Press Oxford.

OECD (2013a). Health expenditure. In *OECD Factbook 2013: Economic, Environmental and Social Statistics*. OECD Publishing.

OECD (2013b). Life expectancy. In *OECD Factbook 2013: Economic, Environmental and Social Statistics*. OECD Publishing.

Oliveira, P. (2012). Users as service innovators: The case of patient innovation. In Lakhani, K. and Harhoff, D., editors, *Revolutionizing Innovation – A book honoring Prof. Eric von Hippel's 70th birthday*. MIT Press.

Oliveira, P. and von Hippel, E. (2011). Users as service innovators: The case of banking services. *Research Policy*, 40(6):806–818.

Oliveira, P., von Hippel, E., and DeMonaco, H. (2011). Patients as healthcare innovators: The case of cystic fibrosis. Working paper, MIT.

Paul, S., Mytelka, D., Dunwiddie, C., Persinger, C., Munos, B., Lindborg, S., and Schacht, A. (2010). How to improve r&d productivity: the pharmaceutical industry's grand challenge. *Nature Reviews Drug Discovery*, 9(3):203–214.

Porter, M. (1990). The competitive advantage of nations. *Harvard Business Review*, 68(2):73–93.

Quinn, J. B. (1985). Managing innovation: controlled chaos. *Harvard Business Review*, 63(3):73–84.

Rao, S., Tang, J., and Wang, W. (2002). The importance of skills for innovation and productivity. *International Productivity Monitor*, 4:15–26.

Rogers, E. M. (2010). *Diffusion of innovations*. Simon and Schuster.

Rothwell, R. (1992). Successful industrial innovation: critical factors for the 1990s. *R&D Management*, 22(3):221–240.

Schilling, M. A. (2005). *Strategic management of technological innovation*. Tata McGraw-Hill Education.

Schmookler, J. (1966). *Invention and economic growth*, volume 26. Harvard University Press Cambridge, MA.

Shah, S. K. and Tripsas, M. (2007). The accidental entrepreneur: The emergent and collective process of user entrepreneurship. *Strategic Entrepreneurship Journal*, 1(1-2):123–140.

Shcherbatiuk, V. (2012). Users as developers and entrepreneurs of medical treatments/devices: the case of patients and their families and friends. Master's thesis, Universidade Católica Portuguesa.

Strull, W. M., Lo, B., and Charles, G. (1984). Do patients want to participate in medical decision making? *JAMA: The Journal of the American Medical Association*, 252(21):2990–2994.

Thomke, S. and von Hippel, E. (2002). Customers as innovators: A new way to create value. *Harvard Business Review*, 80:74–81.

Urban, G. and von Hippel, E. (1988). Lead user analyses for the development of new industrial products. *Management Science*, 34(5):569–582.

Utterback, J. M. (1974). Innovation in industry and the diffusion of technology. *Science*, 183(4125):620–626.

von Hippel, E. (1976). The dominant role of users in the scientific instrument innovation process. *Research Policy*, 5(3):212–239.

von Hippel, E. (1977). The dominant role of the user in semiconductor and electronic subassembly process innovation. *IEEE Transactions on Engineering Management*, EM-24(2):60–71.

von Hippel, E. (1978). Successful industrial products from customer ideas. *The Journal of Marketing*, pages 39–49.

von Hippel, E. (1982). Appropriability of innovation benefit as a predictor of the source of innovation. *Research Policy*, 11(2):95–115.

von Hippel, E. (1986). Lead users: a source of novel product concepts. *Management Science*, 32(7):791–805.

von Hippel, E. (2005). Democratizing innovation: The evolving phenomenon of user innovation. *Journal für Betriebswirtschaft*, 55(1):63–78.

von Hippel, E. (2007). *The sources of innovation*. Springer.

von Hippel, E., Ogawa, S., and Jong, J. P. J. (2011). The age of the consumer-innovator. *MIT Sloan Management Review*, 53(1):27–35.

West, M. A. (1990). *The social psychology of innovation in groups*. John Wiley & Sons.

Wicks, P., Massagli, M., Frost, J., Brownstein, C., Okun, S., Vaughan, T., Bradley, R., and Heywood, J. (2010). Sharing health data for better outcomes on patientslikeme. *Journal of Medical Internet Research*, 12(2).

Wolfe, R. A. (1994). Organizational innovation: Review, critique and suggested research directions. *Journal of Management Studies*, 31(3):405–431.

World Health Organization (2006). *Constitution of the World Health Organization – Basic Documents*. Supplement. Forty-fifth edition.

Appendix A

Complementary and Alternative Medicine

NCCAM[1] defines complementary and alternative medicine (CAM) as "a group of diverse medical and health care interventions, practices, products, or disciplines that are not generally considered part of conventional medicine". As most of CAM therapies are not accepted nor recommended by conventional medicine, much use of its use might represent clear examples of patient's autonomy in treatment decision-making.

The use of CAM increased substantially in the 1990's (Eisenberg et al., 1998) and in 2002 36% of US adults used some form of CAM, and 62% if we include prayer specifically for health reasons (Barnes et al., 2004). This phenomenon led to an urge in conducting scientific tests to prove CAM efficacy. There were, and still are, huge flaws and deficiencies in the design and conduction of randomized controlled clinical trials (Fontanarosa and Lundberg, 1998). This wave contributed to a lot of research in that direction, but although in 2008 more than 7500 CAM trials were indexed in Medline the safety and efficacy of many CAM therapies is still unknown (Chan, 2008).

[1] National Center for Complementary and Alternative Medicine (http://nccam.nih.gov/)

Appendix B

Survey

1 - General Questions.

1. Does anyone in your family suffer from a rare disease?
() Yes, me
() No
() Yes, a close relative or someone in my family (Indicate relationship:)
2. What is the rare disease?
3. For how long has this person suffered from that disease?
4. Is this condition limiting your life?
() No
() Yes
() Don't know/ Prefer not to answer
() N/A
4.1 From 1 to 5 what is the weight that these limitations have in your life?
5. Are there flaws in the existing treatments/therapies/equipment?
() No
() Yes
() Don't know/ Prefer not to answer
() N/A
5.1 From 1 to 5 to what extent are there flaws in the following areas:
Equipment:
Treatments/Therapies:
Behavior/ Habit/ Strategy:
6. Are the expenses with the disease significant?
() No
() Yes

() Don't know/ Prefer not to answer

() N/A

6.1 Can you specify a monthly expense?

7. Have you ever developed, adapted, or used in a way that is different from its original use, a medical equipment, a treatment, or therapy, or a behavior, or habit, or strategy to better cope with the disease?

() No

() Yes, a medical equipment (please describe)

() Yes, a Treatment/Therapy (please describe)

() Yes, a Behavior/ Habit/ Strategy (please describe)

2 - Questions about the innovation.

From 1 (non-existing) to 7 (excellent) how do you rate the quality of life of the patient:

Before the innovation:

After the innovation:

4 - Demographic questions.

28. Birth year of the patient?

29. Gender of the patient?

() Female

() Male

30. Birth year?

31. Gender?

() Female

() Male

32. Country of residence:

() Portugal

() Other:

33. Economic status:

() Student

() Employed

() Unemployed

() Retired

() Looking after home

() Other:

34. Education level?

() Primary
() Secondary
() Bachelor
() Master
() PhD
() Other:

35.Field of study?

() Exact Sciences
() Natural Sciences
() Engineering & Technology
() Agricultural Sciences
() Social Sciences
() Art

36. Marital Status?

() Single
() Married
() Divorced
() Cohabitation
() Widower

37. Number of children?

38. Are you (or a member of your family) member of another rare disease association?

() No
() Yes (If yes, which?):

5 - ICT Related Questions.

39. Do you have access to internet?

() No
() Yes

40. What social networks do you use?

() Facebook
() Social networks in the health area (please specify which):

41. We are developing a platform where patients of chronic and rare disease can share among them experiences and solutions for their pathologies. Would you join such platform?

() No
() Yes

Appendix C

Independent variables

VARIABLE	TYPE OF DATA	MEASUREMENT	UNDERLYING QUESTIONS
Block 1: Demographic variables.			
Age	Categorical	5 Classes: 18-24; 25-34; 35-49 (control variable); 50-64; >64	***
Gender	Dichotomous	***	***
Education	Dichotomous	The dichotomous consists of holding (1) or not (0) a university degree.	1) A radio-button question about the level of education with six possible options: Primary, Secondary, Bachelor, Master, PhD, and Other. **Note:** Though, we are interested in assessing the effect of holding a university degree (Bachelor, Master or PhD).
Marital Status	Categorical	5 Classes: Married (baseline); Single; Divorced; Cohabitation; Widower	1) A radio-button question about the marital status with the five mentioned measurement options.
Professional Situation	Categorical	6 Classes: Employed (baseline); Student; Unemployed; Retired; Looking after home; Other	1) A radio-button question about the professional situation with the six mentioned measurement options.
Block 2: Non-demographic variables.			
Years with the disease	Continuous	***	1) A text-box question inquiring for how long the person had had the disease and/or since what age.
Type of disease: ICD-10 code	Categorical	5 Classes: Congenital malformations, deformations and chromosomal abnormalities; Endocrine, nutritional and metabolic diseases; Diseases of the nervous system; Diseases of the musculoskeletal system and connective tissue; Other (baseline)	1) A text-box question asking to identify the disease. **Note 1:** We manually sorted the disease into the ICD-10 codes. The ICD (International Statistical Classification of Diseases and Related Health Problems) is a medical classification list by the World Health Organization. There are 22 possible classes, of which 15 were found in our sample. As nearly 85% of the diseases belong to only four classes, we created a class, "Other", that gathered the 11 classes with very low frequencies. **Note 2:** All the NA cases are excluded from the model.
Relationship with the patient	Categorical	5 Classes ("the patient is…"): Me; Son; Spouse; Father; Other (baseline)	1) A check-box question asking if the interviewee is a patient or a caregiver. 2) For caregivers only: a text-box to specify the relationship with the patient. **Note:** As nearly 90% of the cases belong to only four classes, we created a class, "Other", that gathered the remaining eight.
Limitations in daily life	Categorical	6-point scale: 1 ("No limitations") to 6 ("Huge limitations")	The 6-point scale is a result of the following questions: 1) A radio-button question asking if the interviewee consideres his life limited, with four options: yes, no, PNA and NA. 2) For those that answered "yes" only: a 5-point Likert scale: 1 ("Few limitations") to 5 ("Huge limitations").
Dissatisfaction with available treatment	Categorical	13-point scale: 1 (Completely satisfied) to 13 (Completely dissatisfied with all types of offered treatment)	The 13-point scale is a result of the following questions: 1) A radio-button question asking if the interviewee considers there are flaws in the available treatments offered, with four options: yes, no, PNA and NA. 2) For those that answered "yes" only: three 5-point Likert scales to specify to what extent 1 ("No limitations") to 5 ("Huge limitations") there are limitations in: 1) Medical equipment, 2) treatments, or therapies, and 3) Behaviors, habits, or strategies. **Note:** We removed three points to the grade due to option 1 "No limitations" in the Likert scales.
Expenses awareness	Dichotomous	The dichotomous consists on the one hand of those that think the expenses with the disease are significant, simultaneously is able to specify a number (1), and the remaining cases (0), except for.	1) A radio-button question asking if the interviewee considers the expenses with the disease significant, with four options: yes, no, PNA and NA. 2) A text-box question to specify the value. In this case we manually sorted the data into the following cases: does know (1), does not know (0), vague, i.e. "a lot" (0), and PNA. **Note:** All the cases of NA and PNA, are excluded from the model.
ICT readiness	Categorical	4-point scale: 1 (No ICT readiness) to 4 (High ICT readiness).	This scale results from attributing 1 point for each "yes" answer in the following three questions: 1) Radio-button question about the use of internet (yes/no). 2) Radio-button question about the use of Facebook (yes/no). 3) Radio-button question about willingness to join a platform where patients share solutions (yes/no). **Note:** As the three component measures are highly correlated (r > .50, except use of facebook and willingness to join the platform, that have r = .36)it is meaningful to consider only one ICT readiness construct (Morrison et al., 2000).

Table C.1: Independent variables measurement.

Appendix D

Characteristics of the sample

DEMOGRAPHIC VARIABLES	COUNT	PERCENTAGE
Age		
Class: 18-24	13	3%
Class: 25-34	95	19%
Class: 35-49	222	45%
Class: 50-64	110	22%
Class: >64	56	11%
Gender		
Female	419	84%
Male	77	16%
Education		
Holds university degree	190	38%
Does not hold university degree	306	62%
Marital Status		
Married	277	56%
Single	95	19%
Divorced	58	12%
Cohabitation	48	10%
Widower	18	4%
Professional Situation		
Employed	252	51%
Student	10	2%
Unemployed	84	17%
Retired	108	22%
Looking after home	30	6%
Other	12	2%

Table D.1: Demographic characteristics of the survey sample. (N=496)

NON-DEMOGRAPHIC VARIABLES	COUNT	PERCENTAGE
Number of years with the disease		
Average	12.4 years	
Standard Deviation	12.8 years	
Minimum	0 years	
Maximum	88 years	
Type of disease: ICD-10 code		
Congenital malformations, deformations and chromosomal abnormalities	154	31%
Endocrine, nutritional and metabolic diseases	73	15%
Diseases of the nervous system	93	19%
Diseases of the musculoskeletal system and connective tissue	78	16%
Other	74	15%
N/A	24	5%
Relationship with the patient		
Me	203	41%
Son	205	41%
Spouse	19	4%
Father/Mother	26	5%
Other	43	9%
Limitations in daily life		
Value = 0	86	17%
Value = 1	4	1%
Value = 2	37	7%
Value = 3	102	21%
Value = 4	136	27%
Value = 5	123	25%
N/A	6	1%
Prefered not to answer	2	0%
Dissatisfaction with available treatment		
Average	5.3	
Standard Deviation	4.2	
Minimum	1	
Maximum	13	
N/A	10	2%
Prefered not to answer	21	4%
Expenses awareness		
Consider expenses significant and can specify a value	173	35%
Don't consider expenses significant and/or cannot specify a value	323	65%
ICT readiness		
Value = 0	42	8%
Value = 1	44	9%
Value = 2	103	21%
Value = 3	307	62%

Table D.2: Non-demographic characteristics of the survey sample. (N=496)